[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Government-Owned Invention; Availability for Licensing

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: The inventions listed below are owned by an agency of the U.S. Government.

FOR FURTHER INFORMATION CONTACT: Licensing information may be obtained by emailing the indicated licensing contact at the National Heart, Lung, and Blood, Office of Technology Transfer and Development Office of Technology Transfer, 31 Center Drive Room 4A29, MSC 2479, Bethesda, MD 20892-2479; telephone: 301-402-5579. A signed Confidential Disclosure Agreement may be required to receive any unpublished information.

SUPPLEMENTARY INFORMATION: The following inventions are available for licensing in accordance with 35 U.S.C. 209 and 37 CFR Part 404 to achieve expeditious

commercialization of results of federally-funded research and development. Technology description follows.

Lentiviral Protein Delivery System For RNA-guided Genome Editing

Description of Technology: This invention provides an HIV-1-based lentiviral vector system for gene correction strategies involving a homologous recombination with a variation of the CRISPR/Cas9 system. Such systems are being explored as potential therapies for certain hereditary diseases. This system comprises (a) a lentivirus vector particle comprising a lentiviral genome which encodes at least one guide RNA sequence that is complementary to a first DNA sequence in a host cell genome, (b) a Cas9 protein, and optionally (c) a donor nucleic acid molecule comprising a second DNA sequence. In addition, the invention provides a host cell comprising the foregoing system, as well as a method of altering a DNA sequence in a host cell comprising contacting a host cell with the foregoing system. Alternatively, the invention also provides a fusion protein comprising a Cas9 protein and a cyclophilin A (CypA) protein, wherein the fusion protein binds to the lentivirus vector particle, as well as a lentiviral vector particle comprising such a fusion protein. Other such lentivirus-based vectors encode a guide RNA, which contains a specific sequence that recognizes a target gene, and a Cas9 endonuclease, which cuts at the specific site. However, such systems present some problems due to constitutive expression of Cas9 endonuclease in lentiviral vector-transduced cells and the large size of the Cas9 gene. The variation of this invention delivers the Cas9 endonuclease directly, instead of the gene encoding the protein.

Potential Commercial Applications: Clinical trials for hereditary diseases such as sickle-cell disease and beta-thalassemia are good market opportunities. Gene correction using the disclosed lentiviral vector system are being tested with respect to the beta-globin gene and the BCL11A gene to treat sickle-cell disease and will be used for induced pluripotent stem cell (iPS) generation.

Development Stage: Early-stage. In vitro data in cell-line models available.

Inventors: Naoya Uchida, Juan J. Haro Mora and John F. Tisdale (NHLBI).

Intellectual Property: US Application No. 62/236,223, filed October 2, 2015 and PCT/US2016/054759, filed September 30, 2016, (NIH Reference No. E-165-2015/0,1).

Publications: Lentiviral protein delivery system for RNA-guided genome editing, PCT Publication No. WO/2017/059241, published April 6, 2017.

Licensing Contact: Cristina Thalhammer-Reyero, Ph.D., M.B.A.; 301-435-4507; thalhamc@mail.nih.gov.

Collaborative Research Opportunity: The National Heart, Lung and Blood Institute is seeking statements of capability or interest from parties interested in collaborative

research to further develop, evaluate or commercialize this technology. For collaboration opportunities, please contact Denise Crooks at crooksd@mail.nih.gov.

Dated: March 22, 2018.

Cristina Thalhammer-Reyero,

Senior Licensing and Patenting Manager,

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National Heart, Lung, and Blood Institute.

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